The REFoRMS Project

Understanding Decision Making for Relapsed and Refractory Rhabdomyosarcoma

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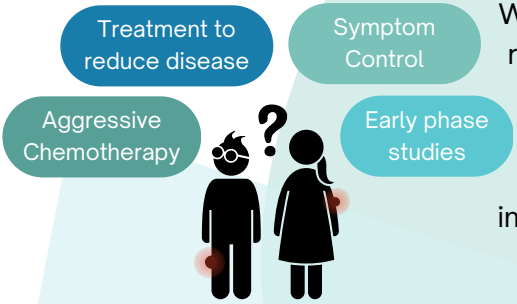
WHAT WAS THE PROBLEM?

Rhabdomyosarcoma is a form of cancer that most commonly affects children and young people. About one third of children and young people with rhabdomyosarcoma will have disease that does not respond to treatment (refractory) or that comes back after treatment (relapsed).



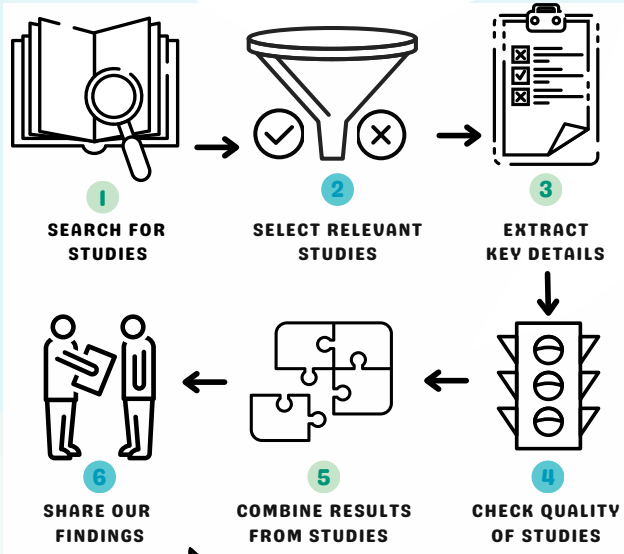
When a child or young person has relapsed or refractory rhabdomyosarcoma, families have to make difficult decisions about what treatment to give next. This could include taking part in an **early phase study**, which investigates new therapies, where not much is known about the drug's safety or effectiveness.

WHAT DID WE DO?

We looked for all early phase studies conducted after 2000 that have investigated treatments for relapsed and refractory rhabdomyosarcoma to see how effective different treatments are, using an approach called a systematic review.

We identified potentially relevant studies by searching online libraries (where research data is stored). We also looked at online registers of studies that researchers say they were going to do (called clinical trial registry).

We found **16,965** possibly relevant studies. Two researchers looked through all of these and found **129** relevant published studies and **99** clinical trials. 63 of these trials were still open to recruit new people to their study.



A summary of the systematic review process

WHAT DID WE FIND?

WHO?

More than **1,100 children and young adults** with relapsed or refractory rhabdomyosarcoma were included.

Eligibility Criteria

Only five studies specifically looked at children and young adults with relapsed or refractory rhabdomyosarcoma

93 studies included different tumour types with relapsed or refractory disease

Age

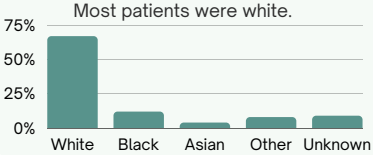
Most studies included patients where the average (median) age was over 10 years. Very few studies included children under 3 years.

Sex

The studies included more males (57.1%) than females.

Ethnicity

Most patients were white.



WHERE?

Most studies were conducted in the USA (62%), but they also took place across Europe, Canada, Japan, Australia/New Zealand, South Asia and North Africa. Some studies recruited children from multiple countries. 10% of studies were conducted in low/middle income countries.

WHAT?

Which treatments were investigated?

Chemotherapy 78

29 single-agent
24 multi-agent
22 novel agents
3 metronomic

Cellular Therapies 6

Targeted Therapies 4

Vaccine Therapies 6

Stem Cell Transplant 5

HOW EFFECTIVE WERE THEY?

SURVIVAL

Not many studies (less than 30) looked at how long children and young people survived after they received treatment.

For those that did, 70% said that the time until the disease progressed was short - on average less than 6 months.

RESPONSE

All of the studies looked at the response rate, which measures whether the tumour has grown or shrunk on a scan. On average, the treatment made the tumour shrink on a scan in 21.6% of children and young adults with rhabdomyorsarcoma. It is not clear whether a 'response' is associated with longer survival.

SIDE EFFECTS

Studies also looked at the side effects of taking these treatments. Most of the side effects resulted in changes to the child or young person's blood tests. The side effects were different, depending on the treatment given.

WHAT WILL HAPPEN NEXT?

We understand that these are really difficult findings for children and young people with relapsed/refractory rhabdomyosarcoma, their families and the people who care for them. Here are our next steps:

We are doing an interview study where we speak to patients and families about how they have made, or are making, decisions about treatment. This will help us understand the decision-making process and how best to support families making these choices.

The results of this systematic review and the interview study will be combined in a best practice statement which will provide advice and support to clinicians and families about important things to consider when discussing treatment options.

We will be sharing our findings with families and professionals, including healthcare teams, researchers and policy makers, so that they can use the information in patient care, and in designing research studies in the future.

We will work with researchers to think about better ways to design and report high quality research that is more helpful to answering these kinds of questions in the future.

We are working on a project called Living-REFoRMS which will provide a regularly updated online resource of information about early phase trials for children and young people with relapsed and refractory rhabdomyosarcoma.

If you would like to know more about REFoRMS or keep updated on its progress, check out our website, or contact us via email or on Twitter.

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